Blindness Fact Sheet

CIRM funds many projects seeking to better understand diseases of blindness and to translate those discoveries into new therapies.

Description

Nearly a million Americans are blind, with another 2.4 million suffering significant visual impairment. While there are several causes of blindness, the leading cause of all visual impairment is age-related macular degeneration, which affects 1.7 million Americans.

California's stem cell agency funds research into potential therapies for three of the causes of blindness. All the research teams are seeking to use various forms of stem cells to rescue or replace cells in the eye damaged or threatened by the diseases. Several groups are working on ways to restore vision for people with age-related macular degeneration (AMD). Other projects are looking to preserve vision in patients with retinitis pigmentosa, and to restore clarity to the surface of eyes impacted by corneal disease.

Macular Degeneration

In AMD the layer of cells that support the photoreceptors is destroyed. Without this support system, the photoreceptors, the cells that actually allow us to sense light start to malfunction. CIRM-funded teams are looking at various methods of replacing this layer of support cells called RPE (retinal pigment epithelial) cells. Some are using embryonic stem cells as a starting point to generate new RPE cells. Others are using stem cells obtained by reprogramming adult cells to be like embryonic cells, which could potentially come from the patients' themselves.

Retinitis Pigmentosa

Retinitis pigmentosa, an inherited and progressive vision loss that leaves most patients legally blind by mid-life, directly destroys the photoreceptors. CIRM-funded researchers are seeking to use stem cells to rescue the receptors from further damage and potentially replace them with new ones.

Limbal Stem Cell Deficiency

The cornea, the outer surface of the eye, is constantly refreshed by stem cells that reside in neighboring tissue. But some people just don't have enough of these stem cells, called Limbal stem cells, to make enough new cornea cells. CIRM-funded researcher are trying to correct this condition, limbal stem cell deficiency, by retrieving the few existing limbal stem cells, and using various techniques to expand them in the laboratory until there are enough cells to rebuild a healthy cornea.

Some projects we fund are trying to take promising therapies out of the laboratory and closer to being tested in people. These Disease Team Awards encourage the creation of teams that have both the scientific knowledge and business skills needed to produce therapies that can get approval from the Food and Drug Administration (FDA) to be tested in people. In some cases, these awards also fund the early phase clinical trials to show that they are safe to use and, in some cases, show some signs of being effective.

Clinical Stage Programs

University of Southern California

This team is using embryonic stem cells to produce the support cells, or RPE cells, needed to replace those lost in AMD. Because these cells exist in a thin sheet in the back of the eye, they are assembling these sheets in the lab by growing the RPE cells on synthetic scaffolds. These sheets are then surgically implanted into the eye. They are testing the human embryonic stem cell-derived RPE cells in a Phase 1/2a clinical trial to treat the advanced dry form of AMD.

- Read more about this project
- Learn more about this clinical trial

University of California, Irvine

For retinitis pigmentosa, the team is using donor tissue to isolate cells that are part way down the path from neural stem cells to adult

eye tissue. These retinal progenitor cells are grown in large quantities in the lab and then injected into the eye. The team suggests the cells could help in two ways. They may be able to protect the photoreceptors not yet damaged by the disease, and they may be able to form new photoreceptors to replace those already lost. The team is testing the safety of transplanting human retinal progenitor cells into patients with RP in a phase 1/2 clinical trial.

- Read more about this project
- Learn more about this clinical trial

jCyte

The same team from UC Irvine is now conducting a Phase 2b clinical trial for retinitis pigmentosa using the same stem cell derived retinal progenitor cell therapy. The trial, which is sponsored by the company jCyte, will test the treatment in a larger patient population to determine whether the treatment is effective at restoring some vision. After finishing patient enrollment, the team will conduct patient follow up studies and collect of all clinical outcome measures.

- Read more about this project

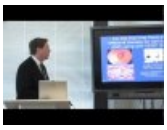
CIRM Grants Targeting Vision Loss

Researcher name	Institution	Grant Title	Grant Type	Approved funds
Karl Wahlin	University of California, San Diego	Microenvironment based optimization of retinal induction using CRISPR-CAS9 reporter pluripotent stem cells as an expandable source of retinal progenitors and photoreceptors.	Inception - Discovery Stage Research Projects	$232,200
Sophie Deng	University of California, Los Angeles	Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy	Late Stage Preclinical Projects	$4,244,211
Henry Klassen	jCyte, Inc	Phase 2b Clinical Study of Safety and Efficacy of Intravitreal Injection of Retinal Progenitor Cells (jCell) for Treatment of Retinitis Pigmentosa	Clinical Trial Stage Projects	$8,295,750

Mark Humayun	University of Southern California	Stem cell based treatment strategy for Age-related Macular Degeneration (AMD)	Disease Team Research I	$18,904,916	
Jeffrey Goldberg	Stanford University	Embryonic Stem Cells for Corneal Endothelial Degeneration	Inception - Discovery Stage Research Projects	$235,836	
David Hinton	University of Southern California	Therapeutic potential of Retinal Pigment Epithelial cell lines derived from hES cells for retinal degeneration.	SEED Grant	$651,607	
Martin Friedlander	Scripps Research Institute	Autologous Retinal Pigmented Epithelial Cells Derived from Induced Pluripotent Stem Cells for the Treatment of Atrophic Age Related Macular Degeneration	Early Translational I	$5,806,321	
Gabriel Travis	University of California, Los Angeles	Development of a Stem Cell-based Transplantation Strategy for Treating Age-related Macular Degeneration	Early Translational I	$5,487,136	
Biju Thomas	University of Southern California	A Novel Tissue Engineering Technique to Repair Degenerated Retina	Inception - Discovery Stage Research Projects	$215,133	
Peter Coffey	University of California, Santa Barbara	Development of Cellular Therapies for Retinal Disease	Research Leadership	$4,850,116	
Henry Klassen	University of California, Irvine	Retinal progenitor cells for treatment of retinitis pigmentosa	Disease Team Therapy Development - Research	$17,144,825	
Deepak Lamba	Buck Institute for Age Research	3D Modeling of Retina using Polymer Scaffolds for Understanding Disease Pathogenesis	Basic Biology IV	$1,212,553	
Kang Zhang	University of California, San Diego	Generation of fibroblast cell lines in patients with common blinding eye diseases	Tissue Collection for Disease Modeling	$1,034,425	
Thomas Novak	Cellular Dynamics International	Generation and characterization of high-quality, footprint-free human induced pluripotent stem cell lines from 3,000 donors to investigate multigenic diseases	hiPSC Derivation	$16,000,000	
Deborah Requesens	Coriell Institute for Medical Research	The CIRM Human Pluripotent Stem Cell Biorepository – A Resource for Safe Storage and Distribution of High Quality iPSCs	hPSC Repository	$9,942,175	
Magdalene Seiler	University of California, Irvine	Restoring vision by sheet transplants of retinal progenitors and retinal pigment epithelium (RPE) derived from human embryonic stem cells (hESCs)	Early Translational IV	$3,998,948	
Mark Humayun	University of Southern California	Phase 1 Safety Assessment of CPCB-RPE1, hESC-derived RPE Cell Coated Parylene Membrane Implants, in Patients with Advanced Dry Age Related Macular Degeneration	Disease Team Therapy Development III	$17,128,661	

Mark Humayun	University of Southern California	Stem cell based treatment strategy for Age-related Macular Degeneration (AMD)	Disease Team Planning	$3,088	
Sophie Deng	University of California, Los Angeles	Regeneration of Functional Human Corneal Epithelial Progenitor Cells	Early Translational II	$697,507	
David Schaffer	University of California, Berkeley	Engineered Biomaterials for Scalable Manufacturing and High Viability Implantation of hPSC-Derived Cells to Treat Neurodegenerative Disease	Tools and Technologies III	$1,239,276	
Sophie Deng	University of California, Los Angeles	Regeneration of Functional Human Corneal Epithelial Progenitor Cells	Early Translational II	$1,524,947	
Shaomei Wang	Cedars-Sinai Medical Center	IND-enabling study of subretinal delivery of human neural progenitor cells for the treatment of retinitis pigmentosa	Late Stage Preclinical Projects	$4,954,514	
Henry Klassen	University of California, Irvine	Human retinal progenitor cells as candidate therapy for retinitis pigmentosa	Early Translational II	$1,803,768	
					Total: $125,607,913.00

CIRM Videos about Vision Loss

 Stem Cell Clinical Trial for Retinitis Pigmentosa: Rosie's Story	 Eyeing Stem Cell Therapies for Vision Loss	 Masayo Takahashi - 2015 Winner of Ogawa-Yamanaka Stem Cell Prize	 A Stem Cell-Based Clinical Trial for Retinitis Pigmentosa: Henry Klassen, UC Irvine
 A Stem Cell-Based Therapy for Retinitis Pigmentosa: The Patient's Perspective	 Hossein Nazari, USC - CIRM Stem Cell #SciencePitch	 Jacqueline Ward, UCSD - CIRM Stem Cell #SciencePitch	 Mark Humayun, USC - CIRM Stem Cell #SciencePitch
Webinar: Focus on the Eye	 Blindness: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting	 Cures through Collaboration: Funding a Team Approach to Disease Research	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Introduction
 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Victoria Jackson	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Michael Yeaman	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Benjamin Greenberg	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Candace Coffee
 Progress and Promise in Macular Degeneration	 Spotlight on Macular Degeneration: Welcoming Remarks	 Spotlight on Macular Degeneration: Seminar by David Hinton, M.D.	 Spotlight on Macular Degeneration: Seminar by Mark Humayun, M.D., Ph.D.
 Spotlight on Macular Degeneration: Seminar by Sharon Hayes	 Stem Cell Based Therapies for Blindness: David Hinton - CIRM Science Writer's Seminar		

News and Information

- *The Stem Cellar*'s entries on macular degeneration research
- Sights on a Cure: Stem cell scientists have macular degeneration in the crosshairs (CIRM)
- Living with Macular Degeneration: Sharon Hayes (CIRM)

Resources

- National Eye Institute: Macular Degeneration Facts
- Find a clinical trial near you: NIH Clinical Trials database
- Macular Degeneration Association
- American Macular Degeneration Foundation
- The Macula Foundation
- Stem Cell Netword eye disease page
- Foundation Fighting Blindness
- Lighthouse for the Blind
- Family Caregiver Alliance
- National Family Caregivers Association

Find Out More:
Stem Cell FAQ | Stem Cell Videos | What We Fund

Source URL: https://www.cirm.ca.gov/our-progress/disease-information/blindness-fact-sheet